4164-01-P

DEPARTMENT OF HEALTH AND HUMAN SERVICES

Food and Drug Administration

[Docket No. FDA-2019-N-0077]

Patient Perspectives on the Impact of Rare Diseases: Bridging the Commonalities; Public Meeting; Request for Comments

AGENCY: Food and Drug Administration, HHS.

ACTION: Notice of public meeting; request for comments.

SUMMARY: The Food and Drug Administration (FDA or the Agency) is announcing a public meeting and an opportunity for public comment on "Patient Perspectives on the Impact of Rare Diseases: Bridging the Commonalities." This public meeting is intended to obtain patients' and caregivers' perspectives on impacts of rare diseases on daily life and to assess commonalities that may help the Agency and medical product developers further understand and advance the development of treatments for rare diseases. Developing a treatment for a rare disease can present unique challenges, such as the small number of individuals affected and heterogenous etiologies and manifestations. While the differences between rare diseases are critically important, it is also important to assess commonalities to synergize product development in rare diseases. The goal of this meeting is to identify common issues and symptoms in rare diseases to help advance medical product development, potentially through the creation of novel endpoints or trial designs that focus on commonalities across a variety of rare diseases.

DATES: The public meeting will be held on April 29, 2019, from 1 p.m. to 5 p.m. The online registration to attend must be received by April 15, 2019. Onsite registration on the day of the

meeting will be based on space availability. Submit either electronic or written comments on the public meeting by May 30, 2019. See the SUPPLEMENTARY INFORMATION section for registration date and information.

ADDRESSES: The public meeting will be held at the FDA White Oak Campus, 10903 New Hampshire Ave., Building 31 Conference Center, the Great Room (Rm. 1503), Silver Spring, MD 20993-0002. Entrance for the public meeting participants (non-FDA employees) is through Building 1, where routine security check procedures will be performed. For parking and security information, please refer to https://www.fda.gov/AboutFDA/WorkingatFDA/BuildingsandFacilities/WhiteOakCampusInformation/ucm241740.htm.

You may submit comments as follows. Please note that late, untimely filed comments will not be considered. Electronic comments must be submitted on or before May 30, 2019. The https://www.regulations.gov electronic filing system will accept comments until midnight Eastern Time at the end of May 30, 2019. Comments received by mail/hand delivery/courier (for written/paper submissions) will be considered timely if they are postmarked or the delivery service acceptance receipt is on or before that date.

Electronic Submissions

Submit electronic comments in the following way:

- Federal eRulemaking Portal: https://www.regulations.gov. Follow the instructions for submitting comments. Comments submitted electronically, including attachments, to https://www.regulations.gov will be posted to the docket unchanged. Because your comment will be made public, you are solely responsible for ensuring that your comment does not include any confidential information that you or a third

party may not wish to be posted, such as medical information, your or anyone else's Social Security number, or confidential business information, such as a manufacturing process. Please note that if you include your name, contact information, or other information that identifies you in the body of your comments, that information will be posted on https://www.regulations.gov.

- If you want to submit a comment with confidential information that you do not wish to be made available to the public, submit the comment as a written/paper submission and in the manner detailed (see "Written/Paper Submissions" and "Instructions").

Written/Paper Submissions

Submit written/paper submissions as follows:

- Mail/Hand delivery/Courier (for written/paper submissions): Dockets Management Staff (HFA-305), Food and Drug Administration, 5630 Fishers Lane, Rm. 1061, Rockville, MD 20852.
- For written/paper comments submitted to the Dockets Management Staff, FDA will post your comment, as well as any attachments, except for information submitted, marked and identified, as confidential, if submitted as detailed in "Instructions."

Instructions: All submissions received must include the Docket No. FDA-2019-N-0077 for "Patient Perspectives on the Impact of Rare Diseases: Bridging the Commonalities." Received comments, those filed in a timely manner (see ADDRESSES), will be placed in the docket and, except for those submitted as "Confidential Submissions," publicly viewable at https://www.regulations.gov or at the Dockets Management Staff between 9 a.m. and 4 p.m., Monday through Friday.

- Confidential Submissions--To submit a comment with confidential information that you do not wish to be made publicly available, submit your comments only as a written/paper submission. You should submit two copies total. One copy will include the information you claim to be confidential with a heading or cover note that states "THIS DOCUMENT CONTAINS CONFIDENTIAL INFORMATION." The Agency will review this copy, including the claimed confidential information, in its consideration of comments. The second copy, which will have the claimed confidential information redacted/blacked out, will be available for public viewing and posted on https://www.regulations.gov. Submit both copies to the Dockets Management Staff. If you do not wish your name and contact information to be made publicly available, you can provide this information on the cover sheet and not in the body of your comments and you must identify this information as "confidential." Any information marked as "confidential" will not be disclosed except in accordance with 21 CFR 10.20 and other applicable disclosure law. For more information about FDA's posting of comments to public dockets, see 80 FR 56469, September 18, 2015, or access the information at: https://www.gpo.gov/fdsys/pkg/FR-2015-09-18/pdf/2015-23389.pdf.

Docket: For access to the docket to read background documents or the electronic and written/paper comments received, go to https://www.regulations.gov and insert the docket number, found in brackets in the heading of this document, into the "Search" box and follow the prompts and/or go to the Dockets Management Staff, 5630 Fishers Lane, Rm. 1061, Rockville, MD 20852.

FOR FURTHER INFORMATION CONTACT: Eleanor Dixon-Terry, Office of Orphan Products Development, Food and Drug Administration, 10903 New Hampshire Ave., Bldg. 32, Rm. 5163, Silver Spring, MD 20933, 301-796-7634, OOPDOrphanEvents@fda.hhs.gov.

SUPPLEMENTARY INFORMATION:

I. Background

The development of drugs, biologics, and devices for rare diseases involves unique challenges. The goal of this meeting is to identify common issues across rare diseases to help address some of these challenges. Rare diseases, often referred to as orphan diseases, are defined based on rarity of occurrence. Although these diseases are individually rare, collectively they are not. According to the National Institutes of Health, there are approximately 7,000 rare diseases affecting an estimated 30 million people in the United States. Many of these rare diseases are serious or life-threatening and many affect children.

The combination of government incentives and scientific advances has fueled extraordinary development in orphan drugs. Since the Orphan Drug Act was passed in 1983, drugs and biologics for over 750 rare disease indications have been developed and approved for marketing. In addition to drugs and biologics, there has been progress in the development of devices for rare diseases. Since 1990, the FDA has approved 74 medical devices for orphan indications under the Agency's Humanitarian Device Exemption program. Despite these successes, we recognize that thousands of rare diseases still have no approved treatments.

Developing a treatment for a rare disease can present unique challenges. Potential challenges include the small number of individuals affected, lack of understanding of the natural history of the disease, phenotypic heterogeneity, and lack of validated endpoints for use in clinical trials. Overcoming these challenges requires collaboration between many stakeholders,

including scientists, product developers, regulators, policy makers, and patients. FDA is committed to working with stakeholders to advance treatment options for patients with rare diseases.

This public meeting will focus on the perspective of those affected by rare diseases. Patients, family members, and caregivers will provide important input on the impact of rare diseases on daily life. While the differences between rare diseases are critically important, this meeting will assess commonalities. The specific goal of this meeting is to identify common issues and symptoms in rare diseases to help advance medical product development, potentially through the generation of novel endpoints or trial designs that focus on commonalities across a variety of rare disease.

FDA will provide a summary document from this public meeting. This meeting will include participants from FDA, the patient community, caregivers, and other interested stakeholders.

II. Topics for Discussion at the Public Meeting

This public meeting will consist of panels of patients/caregivers and facilitated discussions. The aim of the meeting is to hear directly from patients with rare diseases and their caregivers and family members. The meeting will include patients with any rare disease and their caregivers and family members. It is not restricted to a specific rare disease or group of rare diseases.

The meeting will focus on several related topics. Specifically, FDA would like to hear directly from patients with rare diseases and their caregivers and family members about disease symptoms, treatment considerations, and factors relevant to participating in a clinical study or registry. We invite the public to register and participate in our panel discussions. A detailed

agenda and meeting topics will be posted on the following website in advance of the meeting: https://www.fda.gov/NewsEvents/MeetingsConferencesWorkshops/ucm628352.htm.

III. Participating in the Public Meeting

Registration: To register for the public meeting, please visit the following website by April 15, 2019: https://patient-perspectives-rare-diseases.eventbrite.com. Please provide complete contact information for each attendee, including name, title, affiliation, address, email, and telephone.

Registration is free and based on space availability, with priority given to early registrants. Persons interested in attending this public meeting must register by April 15, 2019, 5 p.m. Eastern Time. Early registration is recommended because seating is limited; therefore, FDA may limit the number of participants from each organization. Registrants will receive confirmation when their registration has been received. If time and space permit, onsite registration on the day of the public meeting will be provided beginning an hour prior to the start of the meeting.

If you need special accommodations due to a disability, please contact Eleanor Dixon-Terry, at 301-796-7634, or OOPDOrphanEvents@fda.hhs.gov no later than April 15, 2019.

Requests for Oral Presentations: Patients and patient representatives who are interested in presenting comments as part of the initial panel discussions will be asked to indicate in their registration which topic(s) they wish to address. These patients and patient representatives also must send to Eleanor Dixon-Terry (OOPDOrphanEvents@fda.hhs.gov or 301-796-7634) a brief summary of responses to the meeting topics by April 1, 2019. Details regarding the meeting agenda and topics will be available at https://www.fda.gov/NewsEvents/MeetingsConferencesWorkshops/ucm628352.htm.

FDA will hold an open public comment period to give the public an opportunity to comment. Registration for open public comment will occur in the meeting registration and at the registration desk on the day of the meeting on a first-come, first-served basis.

Panelists and open public comment period speakers will be notified of their selection approximately 7 days before the public meeting. We will try to accommodate all patients and patient representatives who wish to speak, either through the panel discussion, an open public comment period, or audience participation; however, the duration of comments may be limited by time constraints.

Streaming Webcast of the Public Meeting: For those unable to attend in person, FDA will provide a live webcast of the meeting. To register for the streaming webcast of the public meeting, please visit the following website by April 28, 2019: https://www.fda.gov/NewsEvents/MeetingsConferencesWorkshops/ucm628352.htm.

If you have never attended a Connect Pro event before, test your connection at https://collaboration.fda.gov/common/help/en/support/meeting_test.htm. To get a quick overview of the Connect Pro program, visit https://www.adobe.com/go/connectpro_overview. FDA has verified the website addresses in this document, as of the date this document publishes in the *Federal Register*, but websites are subject to change over time.

Transcripts: Please be advised that as soon as a transcript of the public meeting is available, it will be accessible at https://www.regulations.gov. It may be viewed at the Dockets Management Staff (see ADDRESSES). A link to the transcript will also be available on the internet at https://www.fda.gov/NewsEvents/MeetingsConferencesWorkshops/ucm628352.htm.

Dated: February 26, 2019.

Lowell J. Schiller,

Acting Associate Commissioner for Policy.

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